The IDeAI Webinar Series - Introduction

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IDeAI Webinar Series, 2016, Oct 4th

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Welcome

Carl-Frederik Burman

Holger Dette

Ralf-Dieter Hilgers

Malgorzata Bogdan

Mats O. Karlsson

Stephen Senn

Franz König

Geert Molenberghs

France Mentré

Christoph Male

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Who am I?

Ralf-Dieter Hilgers
Head of the Department of Medical Statistics at RWTH Aachen University
Coordinator of the IDeAI Project

- studied mathematics at RWTH Aachen University
- got my PhD at the statistical faculty of the University of Dortmund
- since 2001 head of the Department of Medical Statistics (IMSA) at the Medical Faculty, RWTH Aachen University
- research interest is in optimal design of experiments, randomization procedure and clinical trials
- expertise in teaching, consultation etc.

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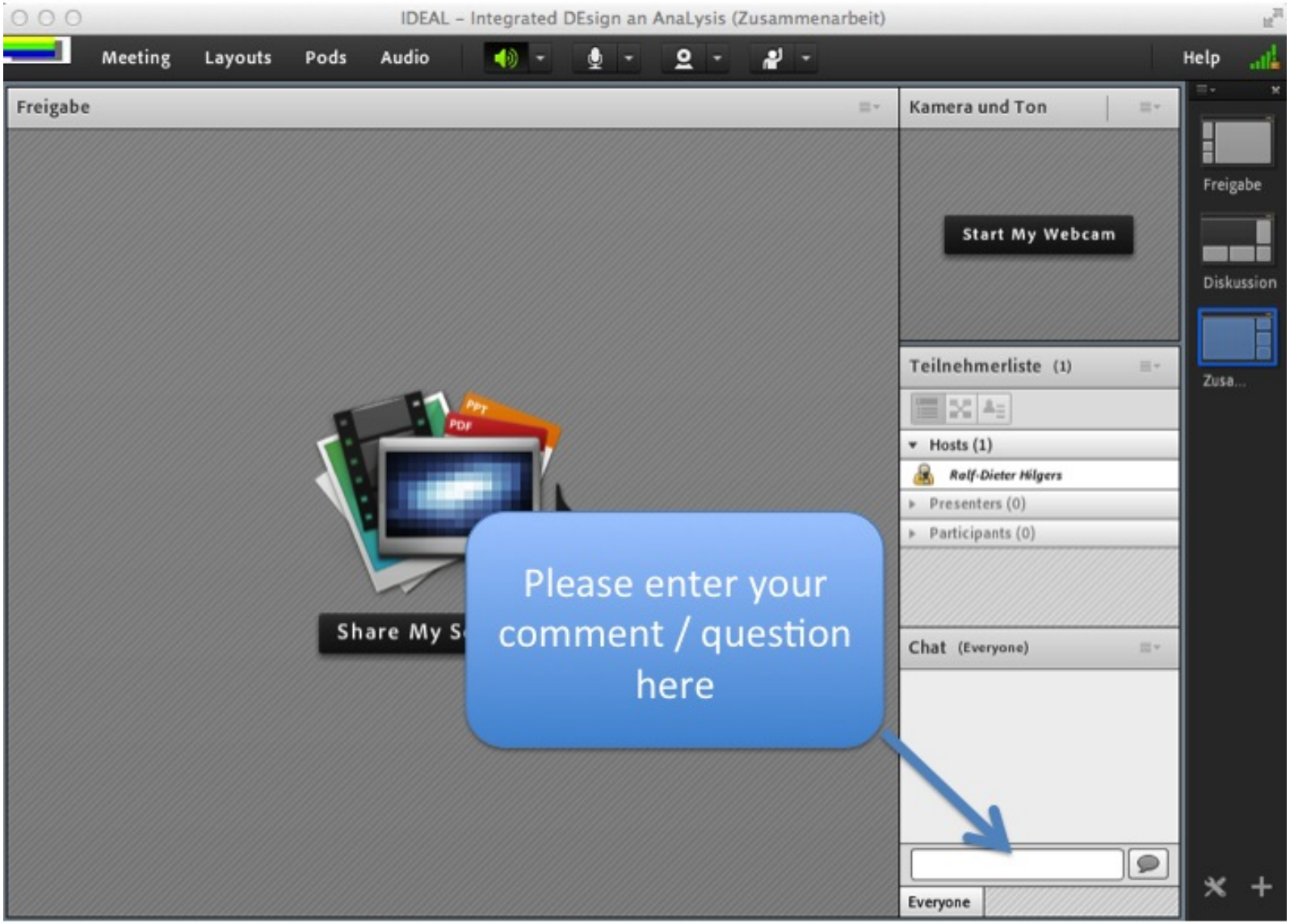
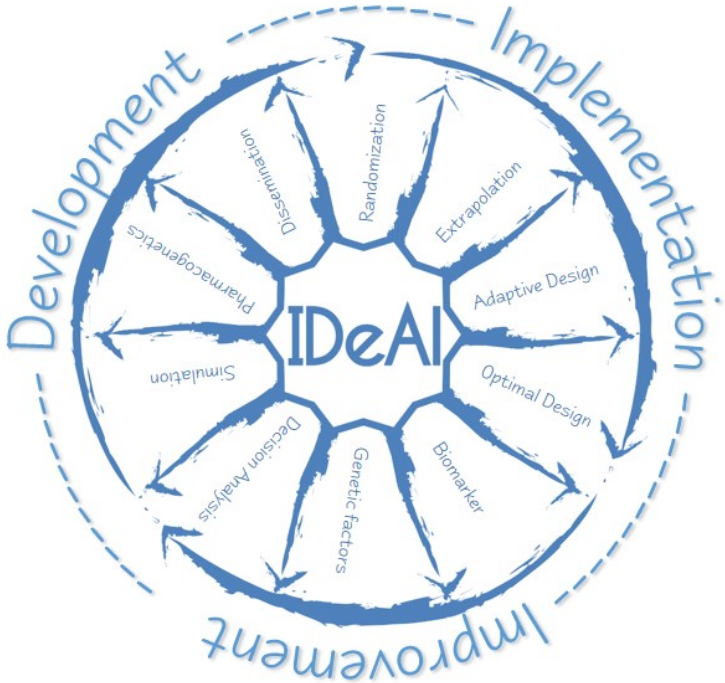
Do you have any Comment?



What is this Webinar about?

- Present an overview of the IDeAI project



- give an overview on the webinar series

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IDeAI Background

There is a pressing need to integrate a broad range of innovative methodologies improving clinical trials in the setting of small sample population groups (SPG).
The objective of this research is to produce methods of general applicability irrespective of indication by Integrated DEsign and AnaLysis of clinical trials in SPG (IDeAI) through a multidisciplinary closely collaborating consortium of researchers from European universities, research institutes and industry.

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EU Funding Initiative

New methodologies for clinical trials for small population groups
FP7-HEALTH-2013-INNOVATION-1.

Objective develop new or improved statistical design methodologies for clinical trials aiming at the efficient assessment of the safety and/or efficacy of a treatment for small population groups in particular for rare diseases or personalised (stratified or individualised) medicine.

Multidisciplinary Framework involve all relevant stakeholders (including industry and patient advocacy groups) as appropriate. Ideally, results would lead to improvement of clinical trial guidelines. Collaboration with relevant organisations outside Europe is welcomed.

Expected Impact Cost efficient clinical trials deriving reliable results from trials in small population groups.

IDeAI - as EU funded project

Integrated DEsign and AnaLysis
of small population group trials

aims to refine the statistical methodology for clinical trials in small population groups by strictly following the concept of an improved integration of design, conduct and analysis of clinical trials from various perspectives.

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IDeAI - People

IDeAI Meeting in Paris, November 2014

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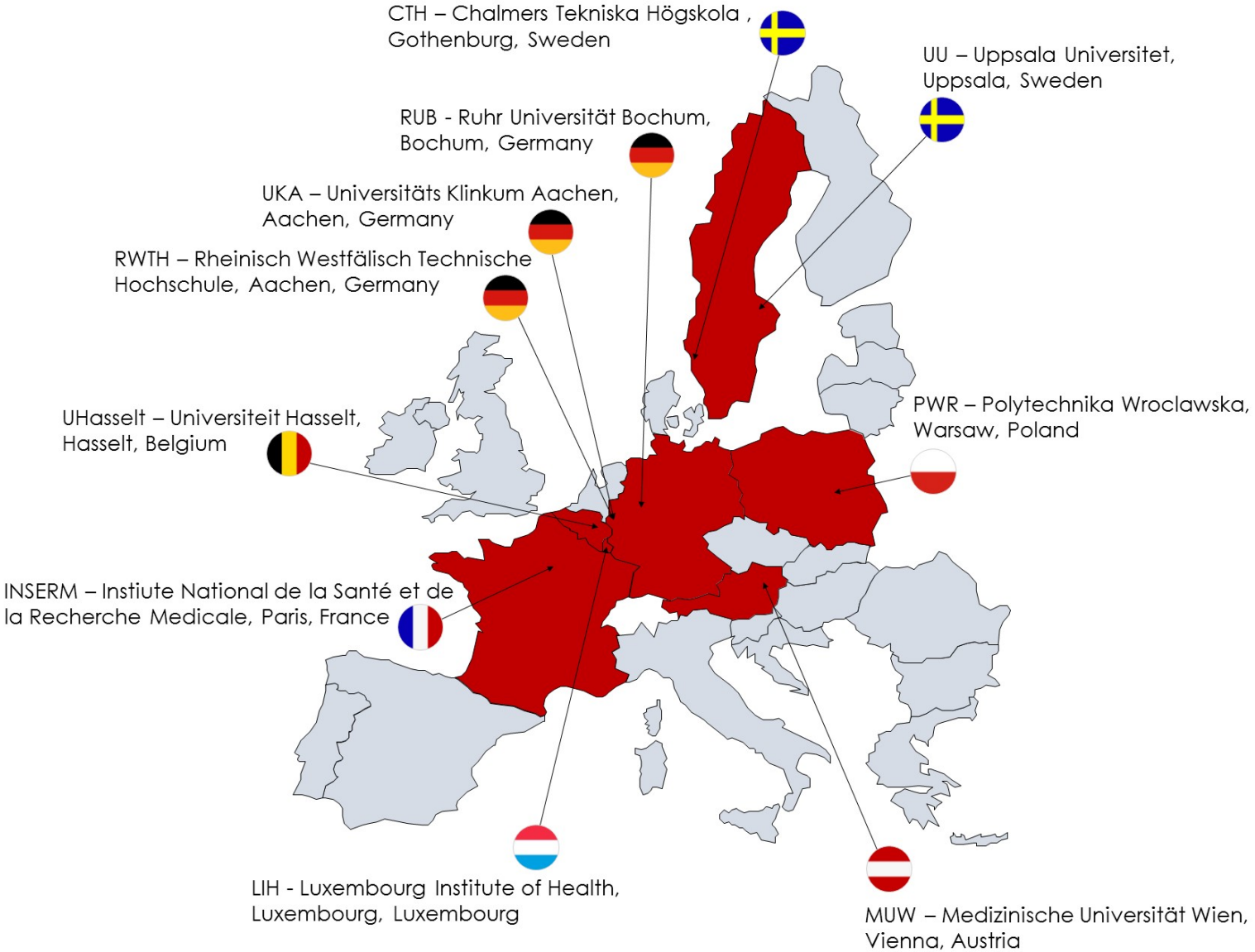
Partner of the IDeAI Project



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IDeAI Work Program

1. **WP 2:** Assessment of randomisation procedures and randomisation based tests in SPG
2. **WP 3:** Extrapolating dose response information to SPG
3. **WP 4:** Adaptive design studies in SPG
4. **WP 5:** Optimal design in mixed models to analyse studies in SPG
5. **WP 6:** Design of pharmacogenetic SPG trials, incl. cross-over trials, n-of-1 trials and enrichment trials
6. **WP 7:** Simulation of clinical trials in SPG
7. **WP 8:** Genetic factors influencing the response to the therapy in SPG
8. **WP 9:** Decision analysis in SPG
9. **WP 10:** Biomarker surrogate endpoints in SPG
10. **WP 11:** Dissemination

External Advisory Board

No	Name	Country
1	Segolene Aymé	(F)
2	Rosemary Bailey	(UK)
3	Paolo Baroldi	(USA)
4	Frank Bretz	(CH)
5	Tomasz Burzykowski	(USA)
6	Martin Forster	(UK)
7	Ralf Herold	(UK)
8	Chris Jennison	(UK)

No	Name	Country
9	Steven A. Julious	(GB)
10	Gerard Nguyen	(F)
11	Paolo Pertile	(I)
12	Gérard Pons	(F)
13	William F. Rosenberger	(USA)
14	Chiara Sabati	(USA)
14	Günther Schmalzing	(D)
14	Gernot Wassmer	(D)

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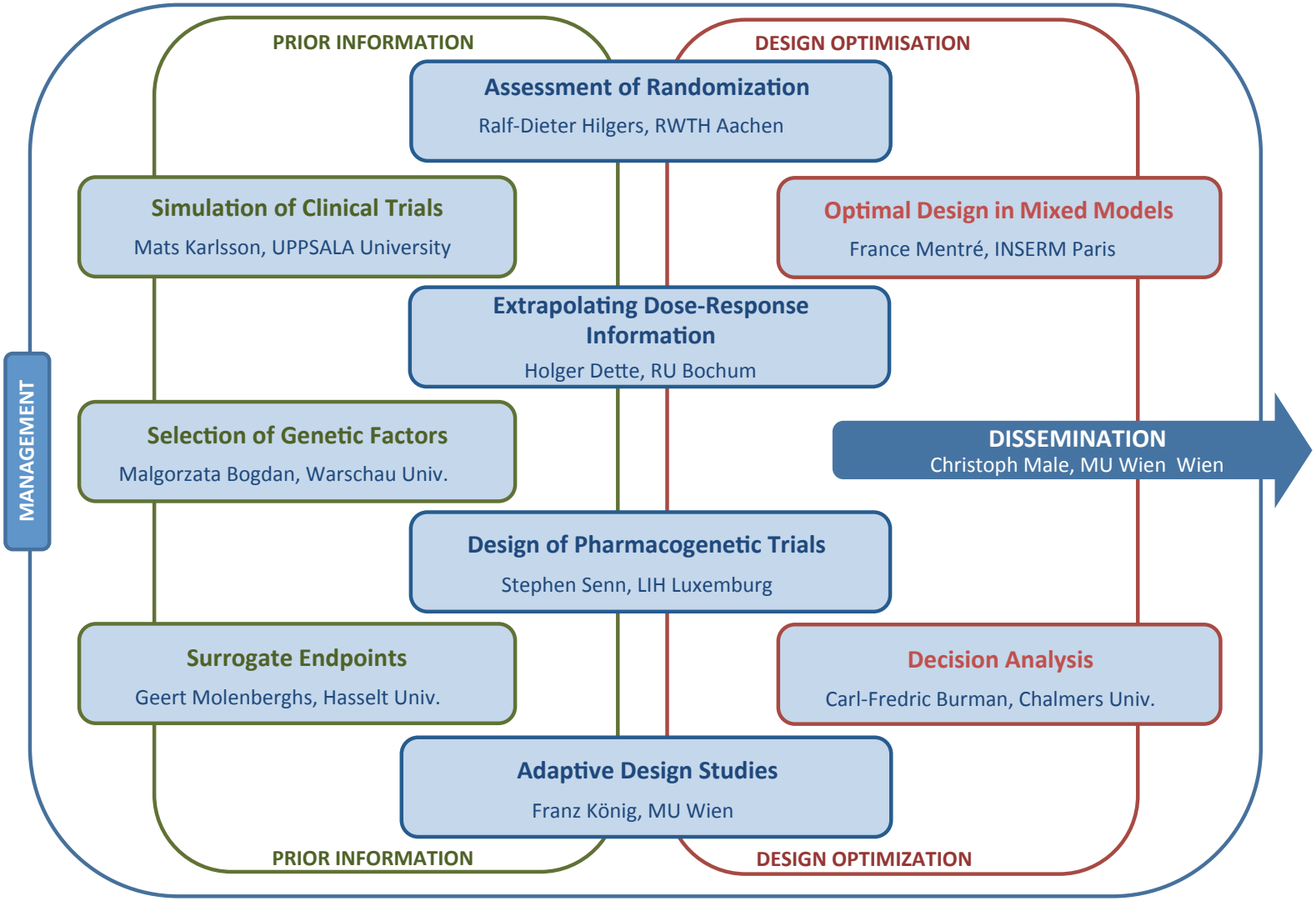
Structure of the IDeAI Project



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EMA issues and interest - IDeAI project(s)

EMA interest	IDeAI - Workpackages
Extrapolation Standards of evidence	WP3: Extrapolating Dose-Response Information (*Holger Dette*) WP 4: Adaptive Design Studies (*Franz König*)
Data-driven decision-making	WP 9: Decision Analysis (*Carl Fredrik Burman*)
Understanding value of research	WP 6: Design of Pharmacogenetic Trials (*Stephen Senn*)
Multidisciplinary simulations	WP 7: Simulation of Clinical Trials (*Mats Karlsson*) WP 5: Optimal Design in Mixed Models (*France Mentré*)
Effects, bias randomisation	WP 10: Surrogate Endpoints (*Geert Molenberghs*) WP 2: Assessment of Randomization (*Ralf-Dieter Hilgers*)

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IDeAI - Schedule

Month											
3	6	9	12	15	18	21	24	27	30	33	36
1. Reporting Period						2. Reporting Period					

6 Milestones

40 Deliverables

IDeAI Output

- 35 publications in peer reviewed journals (actual state)
- presentations at various conferences (among other at the FDA)
- workshops at different conferences
- organized conferences and sessions at conferences
- released various free available software programms
- input to regulatory guidelines
- study stays abroad program
- some input to design and analysis of rare disease clinical trials

IDeAI - Network

- close contact to Kit Roes (asterix) and Nigel Stallard (InSPiRe)
- IRDiRC task force on small population clinical trials
- EMA (e.g, EAB, joint meeting next year)
- DIA small populations working group

asterix

IRDiRC
INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM

InSPiRe
Innovative methodology for small populations research

IDeAI - New Schedule

Month												
3	6	9	12	15	18	21	24	27	30	33	36	42
1. Reporting Period						2. Reporting Period						Prolongation

6 Milestones

40 Deliverables
+ 2 additional

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

InSPiRe
Innovative methodology for small populations research

IDeAI

asterix

What is the Intention of the Webinar Series?

Aim of the IDeAI Series of Webinars

- inform stakeholders about the new IDEAL methodology
- the main achievements of each WP 2 to WP 11 will be presented by the respective work package leader
- the last webinar is a joint presentation of all work packages with the main outcomes of the whole project

What are you may going to expect?

Organisation of the Lectures IDeAI Series of Webinars

- last 1 hour 17:00 to 18:00 CEST
- This webinar series is freely open to anyone.
- The slides of each webinar are published at the public webpage afterwards (www.ideal.rwth-aachen.de).
- Do not hesitate to contact us.

Schedule of the IDeAI Series of Webinars

Date	Presenter	Title
4. Oct	Ralf-Dieter Hilgers	The IDeAI webinar series - introduction
6. Oct	Ralf-Dieter Hilgers	Selection of a randomization rrocedure Does it matter? How it works!
11. Oct	Mats Karlsson	A sampling importance resampling procedure for estimating parameter uncertainty
13. Oct	Holger Dette	Statistical inference for comparing small population groups
18. Oct	Geert Molenberghs	Pseudo-likelihood and split-sample methods in small and very large studies (FP7-IDEAL & ExaScience)

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Schedule of the IDeAI Series of Webinars

Date	Presenter	Title
20. Oct	Franz König	Adaptive level of evidence
25. Oct	Stephen Senn	A little bit me, a little bit you: N of 1 trials, random effects and shrinkage estimators
27. Oct	France Mentré	Using Hamiltonian Monte Carlo to design clinical trials with longitudinal data
10. Nov	Malgorzata Bogdan	Identifying genetic factors influencing important patient's characteristics
15. Nov	Carl-Fredrik Burman	Optimal decisions and stakeholder interactions
17. Nov	Christoph Male Franz König	Dissemination

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Keep in touch with IDeAI

- VISIT THE IDeAI WEBPAGE
  - http://www.ideal.rwth-aachen.de
- Get LinkedIn IDEAL ? FP7 Project
  - http://www.linkedin.com/groups/IDEAL-FP7-Project-6556030
- Twitter @ideal_fp7
  - https://twitter.com/ideal_fp7

The IDeAI Series of Webinars

Enjoy the Webinar Series

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